February 1, 2017

Abeona Therapeutics Enrolls First High Dose Subject in Ongoing Phase 1/2 Gene Therapy Clinical Trial in Sanfilippo Syndrome Type A

- *ABO-102, the leading clinical gene therapy program for Sanfilippo syndrome type A patients, has demonstrated central nervous system (CNS) and peripheral organ disease biopotency*
- *First high dose cohort patient is enrolled, and all patients (n = 4) have cumulative 644 days post-injection with no Serious Adverse Events (SAEs) reported to date*
- *Global ABO-102 enrollments in Europe and Australia commencing in the second quarter*

NEW YORK and CLEVELAND, Feb. 01, 2017 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq:ABEO), a clinical-stage biopharmaceutical company focused on developing therapies for life-threatening rare genetic diseases, announced today that the first high-dose subject was enrolled in the ongoing Phase 1/2 trial for ABO-102 (AAV-SGSH). The first-in-man clinical trial utilizes a single intravenous injection of AAV gene therapy for patients with MPS IIIA (Sanfilippo syndrome type A), a devastating lysosomal storage disease that affects every cell and organ resulting in neurocognitive decline, speech loss, loss of mobility, and premature death.

“The encouraging clinical data from the low-dose patients continue to support a whole-body treatment approach using an intravenously delivered AAV to deliver and drive expression of the SGSH enzyme in all organs of the body, particularly the brain,” stated Kevin M. Flanigan, MD, principal investigator of the clinical trial, Director of the Center for Gene Therapy and Neuromuscular Disorders Program at Nationwide Children’s Hospital and Professor of Pediatrics and Neurology at The Ohio State University College of Medicine. “Additionally, we are encouraged that the 6-month biopotency in two initial subjects are suggestive of sustained disease modification, and we look forward to presenting additional data at the WORLD*Symposium*™ lysosomal storage conference later this month.”

The ongoing Phase 1/2 clinical trial, which has received FastTrack designation by the FDA, is designed to evaluate safety and preliminary indications of efficacy of ABO-102 in patients suffering from MPS IIIA. Per the design of the trial, subjects in the low-dose and high-dose cohorts received a single, intravenous injection of ABO-102 to deliver the AAV viral vector systematically throughout the body to introduce a corrective copy of the gene that underlies the cause of the MPS IIIA disease, particularly the CNS. Subjects are evaluated at multiple time points over the initial 6-months post-injection for safety assessments and initial signals of biopotency.

Previously announced 30-day post-injection data for the low dose cohort indicated:

- ABO-102 reduced GAG (heparan sulfate) in urine 57.6% +/- 8.2%
- ABO-102 reduced GAG (heparan sulfate) in the CSF 25.6% +/- 0.8%
- Reduction in liver volume of 17.7% +/- 1.9%
- Reduction in spleen volume of 17.6% +/- 7.1%

“The combination of CSF and urinary heparan sulfate GAG reduction, liver and spleen volume reduction, and neurological effects support our world leading gene therapy treatment paradigms for patients with MPS III,” stated Timothy J. Miller, Ph.D., President and CEO of Abeona Therapeutics. “We look forward to Dr. Flanigan’s presentation of the biopotency and neurological data at the upcoming WORLD*Symposium*.”

Abeona’s MPS IIIA program, ABO-102, has been granted Orphan Product Designation in the USA and Europe, and has also received the Rare Pediatric Disease Designation in the United States.

About ABO-102 (AAV-SGSH): ABO-102 is an adeno-associated viral (AAV)-based gene therapy for MPS IIIA (Sanfilippo syndrome), which involves a one-time delivery of a normal copy of the defective gene to cells of the central nervous system (CNS) with the aim of reversing the effects of the genetic errors that cause the disease. ABO-102 has been well tolerated in initial subjects of the low-dose cohort with no safety or tolerability concerns identified through 30 day post-injection in patients suffering from MPS IIIA, or Sanfilippo syndrome Type A, a rare autosomal recessive disease caused by genetic mutations that result in a deficiency of SGSH enzyme activity, leading to abnormal accumulation of GAG (specifically, heparan sulfate) in the CNS and systemic tissues and organs. This accumulation of heparan sulfate results in neurocognitive decline, speech loss, loss of mobility, and premature death. Encouraging signs of early biopotency have been observed in urinary and CSF GAG (glycosaminoglycan, specifically, heparan sulfate) measurements, as well as potential disease-modifying effects in the liver and spleen of the initial subjects enrolled and treated in the trial. The clinical study is supported by neurocognitive evaluations, biochemical assessments and MRI data generated in a 25-subject MPS III Natural History Study, also conducted at Nationwide Children's Hospital, where patients continued through one-year of follow up assessments.

About Abeona: Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene therapies for life-threatening rare genetic diseases. Abeona's lead programs include ABO-102 (AAV-SGSH) and ABO-101 (AAV-NAGLU), adeno-associated virus (AAV) based gene therapies for Sanfilippo syndrome (MPS IIIA and IIIB, respectively). Abeona is also developing EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), EB-201 for epidermolysis bullosa (EB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL), ABO-202 (AAV-CLN1) gene therapy for treatment of infantile Batten disease (INCL), and ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder and ABO-302 using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases. In addition, Abeona has a plasma-based protein therapy pipeline, including SDF Alpha™ (alpha-1 protease inhibitor) for inherited COPD, using its proprietary SDF™ (Salt Diafiltration) ethanol-free process. For more information, visit www.abeonatherapeutics.com.

This press release contains certain statements that are forward-looking within the meaning

of Section 27a of the Securities Act of 1933, as amended, and that involve risks and uncertainties. These statements are subject to numerous risks and uncertainties, including but not limited to continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, the impact of competition; the ability to develop our products and technologies; the ability to achieve or obtain necessary regulatory approvals; the impact of changes in the financial markets and global economic conditions; and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and other reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligations to make any revisions to the forward-looking statements contained in this release or to update them to reflect events or circumstances occurring after the date of this release, whether as a result of new information, future developments or otherwise.

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Source: Abeona Therapeutics Inc